SPRING 2023

THE CMTA REPORT

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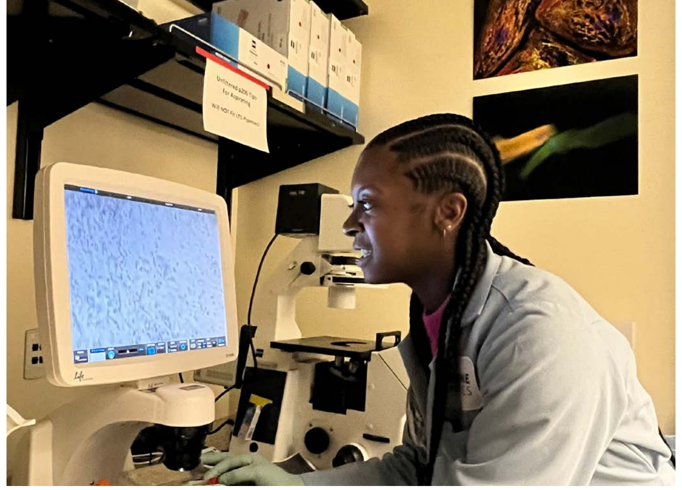
SAILING SOLO AROUND THE GLOBE WITH CMT



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THE CMTA REPORT | SPRING 2023

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Jenny Decker plans to begin her adventurous journey in May, starting from Kona, Hawaii.

NURSE WITH CMT TO CIRCUMNAVIGATE GLOBE SOLO IN SAILBOAT

Nurse Jenny Decker is only 38, but she hears her internal clock ticking faster and faster to the day CMT will curb her ability to live an adventurous life—just as it did her mother.

So, before she can't, Decker wants to knock off the biggest item on her bucket list: circumnavigating the globe on a sailboat. And while it's an amazing personal adventure, she says raising awareness of CMT and funds for research are equally important to her.

She's planning to do it solo, although she will have a first mate: Romeo, her six-pound Maltese Yorkie.

It's an epic journey years in the making. Life has always been a challenge for Decker, who grew up in St. Louis, Mo. and now lives in Kona, Hawaii. She didn't walk until she was 4. Some doctors thought she might never walk, but her life changed after having pro bono surgery on her legs at the Shriners Hospital in St. Louis.

Jenny learned to walk without aids or what she jokingly calls "Forest Gump braces," but she was always clumsier than other kids. Hand tremors, tripping and falling were a regular part of her life. Except for swimming, school sports were out.

Decker was misdiagnosed until age 19 when her mother found out her own medical problems were caused by CMT. She and her mom both had high arches, a classic sign of the disease. Decker knew then she also had CMT. She got a nursing degree and did her best to live her life to the fullest. But as her mother's disease progressed, so did Jenny's desire to cram in a lifetime of adventure before her window of opportunity closed.

That's why in 2016, she became the first person to circumnavigate Hawaii's Big Island solo—in a kayak (Summer 2016 *CMTA Report*, p. 3, bit.ly/3lcUXjf). She paddled clockwise more than 300 miles in 20 days, including a day when swells of 10 to 12 feet and winds at 20 to 25 knots warranted a small craft advisory. She worried about being pushed out to sea, never to be seen again. When she spent the night on shore, she had to swim back to her kayak before dawn to get her daily 12 to 14 hours of paddling in.

continued on page 4

SAILING SOLO WITH CMT

continued from page 3

The great kayak adventure was just a warm-up for what came next. In 2017, while walking around Kona Harbor, she promised herself that she would start sailing around the world by 2020. The only problem was that she had never lived on a boat or sailed one off the coast.

To try out the onboard life, she took a job on a commercial fishing boat based in Kodiak, Alaska. It wasn't easy going.

She was permanently exhausted from 16- to 18-hour workdays, mostly while wet, with no TV, WiFi, running water or bathroom. Life on the boat was austere and grueling, but after the 100-day fishing stint was over, Decker reflected, "I'm not saying I loved it the whole time, but I have amazing footage of using a bucket [to do her business] in the back of the deck while orca whales are coming up."

She returned to Alaska for a second commercial fishing stint on a nicer boat with a bathroom. She also returned because the money was exceptional, allowing her to pay off all her debts.

Decker then returned to the Big Island as a traveling nurse at Kona Community Hospital. In Kona, she joined a sailing club and sailed with friends who owned boats to learn everything she could about the onboard lifestyle.

In early 2020, she was ready to begin her dream of sailing around the world. She sold everything she owned in Hawaii, bought a van on the mainland and began searching for the perfect sailboat in Florida.

Photo by Dustin Reynolds, "The Singlehanded Sailor"

She decided on a vessel called "Made of the Sea" and sailed with a mate from Lake Worth for the Bahamas on March 12, 2020, for what she thought would be an epic journey. It only lasted one country. "We checked into the Bahamas days before the whole world shut down," she said.

They spent 3½ weeks on a deserted island with no contact with the outside world. When they showed up at Green Turtle Cay in the Abacos, Bahamian officials told them they couldn't move their vessel because of the COVID-19 pandemic. For 2½ months they were stranded at sea. Fortunately, they had three months of provisions. When they were allowed to leave, the Atlantic hurricane season was upon them, so they sailed back to the United States, which "fortunately let us back in," Decker said.

Jenny and her first mate, Yorkie Romeo

They stored the boat and Decker went to St. Louis for a crisis COVID-19 nursing assignment. Working on her feet for five 12-hour night shifts a week, doing the emotionally draining work of dealing with dying COVID patients, would be tough for anyone, but especially for Decker with her neurological problems. She was in a lot of pain, but it was lucrative work, and she needed the money to finance her dream.

In 2021, Jenny was ready to start the big trip again, but she wasn't sure she knew enough about sailing solo. She debated for a long time and then called her friend Dustin Reynolds. He was about to set a world record as the first double amputee to circumnavigate the world solo in a sailboat.

Reynolds, who was nearing the end of his own sailing journey, told her, "You already have more experience than I did when I left.…You can do it."

Decker set off again. It began well, and with every 100 miles, her confidence grew. But the trip ended when the masts responsible for hoisting the sails broke. Decker was stranded in the Atlantic, left wondering if the "dismasting" was the universe trying to tell her something.

Reynolds said the boat just wasn't right for her. As it happened, he had the right boat—the Bristol 36 he sailed around the world. He pointed out that Decker could be the second person to set a world record in that boat, as well as the first person with CMT to sail around the world solo.

"Dustin also said, 'Hawaii is where your heart is. Where you should start and finish. Where you learned everything about the ocean.' It just seemed right," Decker said.

> **FOLLOW JENNY'S PROGRESS on Facebook, Instagram or TikTok at @justalap, a name based on Jenny's method of breaking up the trip into achievable goals. Because the trip is such a huge endeavor, Jenny tells herself, "It's just a simple lap" around the world.**

She flew back to Kona in May 2022, bought a 35½-foot live-aboard sailboat and began working on some needed repairs and upgrades, including a new $10,000 engine. To get used to the boat, Decker took a 21-day round trip to the Palmyra Atoll. Reynolds went with her to show her the ropes.

Decker's disease makes her unsteady on land, and it was especially difficult to navigate the deck of a rocking boat. She always wears a harness and straps in to prevent going overboard. "I also crawl, with no shame," she said.

Their 1,800-mile-plus trip was tough, with relentless squalls, 40-knot winds and periods without wind. Things were always breaking. Decker learned diesel engine 101, plumbing and how to figure out how to fix anything that stops working. For a person with a disease that makes turning a screwdriver difficult, it's extra taxing and time-consuming work.

Decker said being able to spend extended time with Reynolds to get familiar with the boat and get his advice was invaluable. She will start her third attempt to circumnavigate the globe in May when a good weather window opens up. She plans to start and finish in Kona.

But first she needs to raise the money to get the boat in tip-top shape. She has a long list of pricey boat repairs that are needed and she'll need money for the journey itself, which she expects will take three to five years. At Reynolds' suggestion, she set up a GoFundMe page for both financial and emotional support at www.gofundme.com/f/just-a-lap.

"I want to continue inspiring those with this ailment, or any disability for that matter," Decker said. "I truly believe you can do anything you put your mind to. So this journey is to inspire all individuals to challenge themselves, set goals, go for them, and share all triumphs or heartaches.…It is what makes us feel the most alive as humans and connects us."

She also wants to do it for herself. "One day I am going to be physically dependent on others," she said. "I know that it's coming. I see what happened to my mother and her quality of life and pain.…I'm doing it now so when I am sitting around in a wheelchair, I can tell some really good stories," she said.

This article was adapted from a piece by Cammy Clark, an editor/news reporter with Maui Now, Big Island Now, Kaua'i Now and the Pacific Media Group and printed here with their permission. Photos courtesy of Jenny Decker. Cover photo and page 3 photo by Lia-Lucine Cary.

CMTA-FUNDED STUDY DEVELOPS FIRST NEW MOUSE MODEL OF CMT1X IN 20 YEARS

A paper published in the February 2023 issue of Experimental Neurology presents the results of a CMTA-funded study that resulted in new mouse models of CMT1X. The previous model was developed over 20 years ago and replicated only a small number of the mutations that cause CMT1X.

The new project was led by Dr. Charles Abrams (University of Illinois at Chicago) and Dr. Steven Scherer (University of Pennsylvania). The team created and characterized additional mouse models of CMT1X that faithfully represent its disease-causing properties. This will support preclinical work for developing treatments.

According to the paper, investigators used CRISPR/Cas9 to develop new "knock-in" models of CMTX1 that are more representative of the spectrum of mutations seen with CMTX1 than the Cx32 knockout (KO) mouse model used previously.

Mice with four genotypes—wild-type, Cx32KO, p.T55I, and p.R75W—were compared. Sciatic motor conduction velocity slowing was the most robust electrophysiologic indicator of neuropathy, showing reductions in the Cx32KO by three months and in the p.T55I and p.R75W mice by six months. At both 6 and 12 months, all three mutant genotypes showed reduced fore-limb and hind-limb grip strength compared to wild-type mice. Performance on six- and 12-mm width balance beams revealed deficits that were most pronounced on the six-mm balance beam at 6 months of age.

In addition, researchers found pathological changes of myelinated axons in the femoral motor nerve in all three mutant lines by 3 months of age, which became more pronounced at 6 to 12 months of age.

According to the authors, sensory nerves (femoral sensory and the caudal nerve of the tail) appeared normal at all ages examined. The results demonstrate that mice can be used to show the pathogenicity of human GJB1 mutations, they concluded.

UNDER THE MICROSCOPE

*In "Under the Microscope," **CMTA Chief Research Officer Katherine Forsey, PhD,** takes a closer look at issues related to the CMTA's Strategy to Accelerate Research. Katherine is a research biologist by training and holds a doctorate in biology from the University of York in the area of expression, activity and localization of metabolic enzymes during preimplantation embryo development. She and members of her family have CMT1A. She lives in Yorkshire, England with her husband and two children.*

More than **$23.5 Million** has been invested in CMTA-STAR research since 2008.

Patients as Partners IN RESEARCH

The CMTA launched the "Patients as Partners in Research" (PPR) initiative in 2018 with the goal of engaging the patient community to participate in its Strategy to Accelerate Research (STAR).

PPR works to:

- **Support access** to surveys, natural history studies and clinical trial opportunities.
- **Educate patients** about research progress in their own subtype and relevant cross-type initiatives.
- **Collect patient-informed data** to improve our support provision.
- **Collate, analyze and present anonymized data** to inform current research partners, the wider CMT research community and the PPR community.

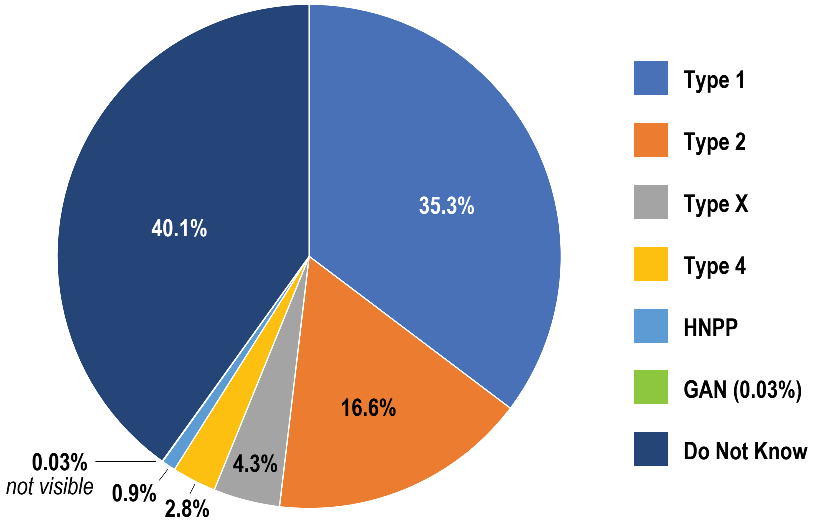
More than 6,200 PPR participants have created profiles via a secure online form as of January 2023, a hugely powerful collection to inform and advance CMT research.

> More than 6,200 PPR participants have created profiles via a secure online form as of January 2023, a hugely powerful collection to inform and advance CMT research.

The profile currently asks 22 questions on topics that include CMT type and subtype, age, location, age of symptom onset, age of diagnosis, genetic testing and Center of Excellence attendance. The CMTA uses the information to share updates with participants personalized to their CMT type and subtype, as well as opportunities to enroll in upcoming clinical trials, natural history studies and researcher-created surveys. Participation in these activities helps researchers and clinicians learn more about how CMT progresses.

The data has been fully anonymized and statistically analyzed to help increase knowledge around, for example, CMT patient type and subtype distribution, the size of the diagnostic gap (number of participants who don't know their CMT type or subtype) and the percentage of people accessing genetic testing.

Patients as Partners in Research — CMT Type Data



Of the 97 percent of patients who responded to the question about their CMT type, just over 40 percent said they don't know their type, proof of a significant diagnostic gap.

More than 4,000 participants have also provided subtype data, representing 47 different CMT subtypes ranging from the most common to the very rare. Some 98 percent of people with Type 1 know their subtype, compared to the 80 percent of Type 4 patients. And just 60 percent of people with Type 2 CMT know their subtypes.

The last finding highlights the importance of the CMTA's Type 2 gene discovery efforts and demonstrates the need for advanced research diagnostic capabilities. Stephan Zuchner, MD, PhD, at the University of Miami is leading the charge to identify new CMT causative genes in addition to the more than 120 already identified. Commercially available genetic testing panels are regularly updated to include the new subtypes. Patients can speak to their neurologists or family doctors for a referral to a genetics counselor for testing, which usually involves taking a family history and a simple blood draw.

Knowing and understanding one's subtype is particularly important as new clinical trials arrive, along with the potential rollout of subtype-specific new treatments for CMT. For example, the gene therapy approaches currently in development through CMTA-sponsored research projects will only work on the specific CMT subtypes they are designed to treat, like the CMT4C project underway with Kleopas Kleopa, MD, at the Cyprus Institute, and the CMT2A and CMT2E CRISPR projects with Bruce Conklin, MD, and Luke Judge, MD, PhD, at the Gladstone Institutes USA. The takeaways from these projects will also benefit all other subtypes.

> Knowing and understanding one's subtype is particularly important as new clinical trials arrive, along with the potential rollout of subtype-specific new treatments for CMT.

The CMTA is also working closely with STAR Alliance partners like ToolGen, which created a potential therapy for CMT1A using CRISPR gene editing technology.

The CMTA plans to expand PPR even further, adding more questions to help researchers understand the CMT patient population better and including essential diversity-capturing questions that are important for future clinical trials and natural history studies. Work is also needed to ensure that traditionally underrepresented groups, which have huge potential to strengthen our knowledge and understanding of CMT, are supported to participate.

When members of the community ask what they can do to help CMT research, one of the things I always suggest is signing up to be a PPR participant. Filling out a profile for oneself or one's child takes less than 10 minutes—asking affected family members to do it takes even less.

To view the current surveys, natural history studies and clinical trial opportunities for Patients as Partners in Research, visit bit.ly/3XSyshQ.

6,000 PROFILES

32 ACTIVE SPONSORED RESEARCH PROJECTS

18 preclinical studies worth over $2 million

JOIN THE PROJECT!

To create your own PPR profile and be part of this important initiative to advance CMT research, visit bit.ly/3x3euW1.

OR SCAN THIS QR CODE

CMTA Co-Sponsors Tour of Conklin Lab And Its 'Life-Changing' Gene Editing Work

Members of the CMTA community toured Dr. Bruce Conklin's lab at Gladstone Institutes in San Francisco during "CMT/Peripheral Neuropathy Day" on Jan. 18. Conklin, a renowned geneticist, is a member of the CMTA's STAR Advisory Board. His lab focuses on biomedical applications of CRISPR gene editing technology, emphasizing motor neuron diseases including CMT.

Co-sponsored by the CMTA, the event also included educational sessions about the future of gene therapy to treat CMT. Gladstone Institutes President Deepak Srivastava, MD, gave an overview of the organization, whose mission is to drive a new era of discovery in disease-oriented science and to mentor tomorrow's leaders in an inspiring and diverse environment.

Luke Judge, MD, PhD, and graduate student Gokul Ramadoss gave a patient-centric presentation on the lab's work and discussed the evolution of gene therapy for neurological diseases. According to them:

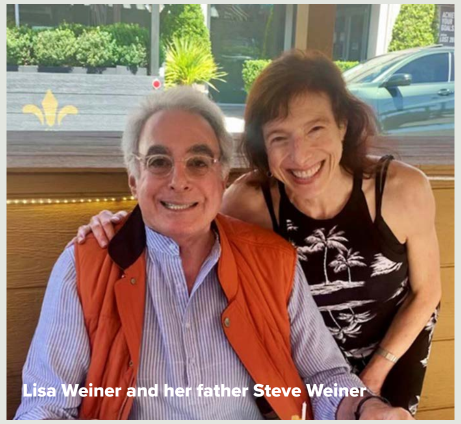
- CRISPR-Cas9 gene editing has the potential to treat many forms of CMT by correcting the root cause of the problem at the genetic level.
- Induced pluripotent stem cells (iPSCs) generated from blood draws allow researchers to test these therapies on nerve cells grown from actual CMT patients.
- The lab has already demonstrated that cells from CMT2E patients are healthier after gene editing, and it is now developing new approaches to treat the largest number of patients.
- Safe and effective methods to deliver CRISPR-Cas9 to the spinal cord without causing long-term side effects will be critical to translating this approach into clinical development.

After the presentation, four panelists took questions from the audience—Judge; UCSF Center of Excellence director Alexander Fay, MD, PhD; CMTA Board Chair Gilles Bouchard; and Zach Nevin, PhD. Conklin moderated the Q&A.

Dr. Bruce Conklin moderated a Q&A session.

It was a memorable afternoon of CMT education and community, and an amazing opportunity to tour a CRISPR lab and meet the brilliant researchers working tirelessly to find a treatment for CMT.

The CMTA and Conklin Lab teams



Lisa Weiner and her father Steve Weiner

THE CONKLIN LAB TOUR:

A Patient's Perspective

Entering the spacious Gladstone Institutes building, my dad, his partner and I mingled with many other CMT warriors, family and friends during a momentous afternoon. The smart, talented and ambitious scientists in Dr. Bruce Conklin's lab opened their doors to explain how their research could help treat CMT and other debilitating neurodegenerative diseases.

Our tour guide, a young PhD graduate and full-time scientist, showed us where they run cells and neurons created from iPSC cells through expensive high-tech machines, then examine them under a microscope at extremely high magnification. We actually saw live pulsing neuron cells, created right there in their lab.

The Conklin Lab is working on several different CMT variants, including the most common, CMT1A. After viewing the lab equipment, the live neuron cells and the numerous refrigerators safeguarding the research, we headed to the building's theater for an hour-long presentation.

Dr. Fay, a pediatric neurologist, explained how spinal muscular atrophy (SMA), which previously ended an infant's life at age 1 or 2, can be eliminated when gene therapy is applied at birth, ideally before symptoms ensue.

It was a remarkable, life-changing afternoon as Gladstone helped us connect the dots.

— Lisa Weiner

EVERYLIFE
FOUNDATION
FOR RARE DISEASES

APPLICATION PERIOD FOR $5,000 SCHOLARSHIPS FOR STUDENTS WITH RARE DISEASES OPENS

The Everylife Foundation for Rare Diseases—with the support of Horizon Therapeutics—will award $5,000 scholarships to 35 recipients with rare diseases in 2023. The application period opens March 8 and runs through April 13. Recipients will be notified in the summer, and funds will be paid directly to their schools in early August 2023.

Anyone 17 or older who is a U.S. resident and who has been diagnosed by a physician with any form of rare disease can apply for a #RAREis Scholarship. Applicants will be asked to provide a Diagnosis Verification Form. Applicants must plan to enroll full-time or part-time in undergraduate or graduate study at an accredited two- or four-year college, university or vocational-technical/trade school for the fall 2023 semester. There is no minimum credit-hour requirement to be part-time and students do not need to be pursuing an undergraduate or graduate degree.

As the foundation's website explains, a disease is defined as rare when it affects fewer than 200,000 people in the United States. CMT meets that definition.

Applicants must provide their latest official transcripts and answer an essay question. In 2022, the question was "How will your education and career goals help you become an advocate for the rare disease community?" Recipients will be selected based on their essay responses, leadership and participation in school and community activities, work experience, academic performance and financial need.

The Everylife Foundation is a 501 (c)(3) nonprofit dedicated to empowering the rare disease patient community to advocate for impactful, science-driven legislation and policy that advances the equitable development of and access to lifesaving diagnoses, treatments and cures. As the foundation website states: "We do not speak for patients. We provide the training, education, resources and opportunities to make their voices heard. By activating the patient advocate, we can change public policy and save lives."

FOR MORE INFORMATION, visit everylifefoundation.org/rare scholarship/.

2023 ‘PIVOTAL’ YEAR FOR PXT3003 TREATMENT FOR CMT1A AS MANUFACTURING SHIFTS TO U.S.

CMTA partner Pharnext announced Jan. 26 that it has completed the manufacturing transfer of PXT3003 from Colomiers, France, to Rochester, N.Y., where it will scale up U.S. production.

CMTA Chief Research Officer Katherine Forsey said the move is good news for U.S. patients, as manufacturing was previously done only in France. “It also shows willingness to provide a more convenient and flexible form on the treatment for CMT1A patients to use,” she added.

According to the announcement, Pharnext is now able to manufacture batches of up to 3,500 liters of PXT3003 oral solution, potentially enough for a commercial supply chain of the treatment.

PXT3003 is a novel, fixed-dose synergistic combination of baclofen, naltrexone and sorbitol formulated as an oral solution and taken three times a day. The three individual components were selected to downregulate the overexpression of PMP22 protein, leading to the improvement of neuronal signaling in dysfunctional peripheral nerves.

The announcement comes just two weeks after Hugo Brugière, the new chairman and CEO of Pharnext, wrote shareholders that the upcoming year will be “pivotal” for PXT3003. The company expects to announce the topline results from its Phase III clinical study of PXT3003 in December. If positive, the results would form the basis of the marketing authorization applications submitted to the U.S. Food and Drug Administration in 2024 and then to the European Medicines Agency at the end of 2024 or the beginning of 2025. If those applications result in marketing authorizations, PXT3003 would be the first treatment specifically approved for patients with CMT1A, bringing hope to hundreds of thousands of patients who suffer from this rare and debilitating peripheral neuropathy, Brugière wrote.

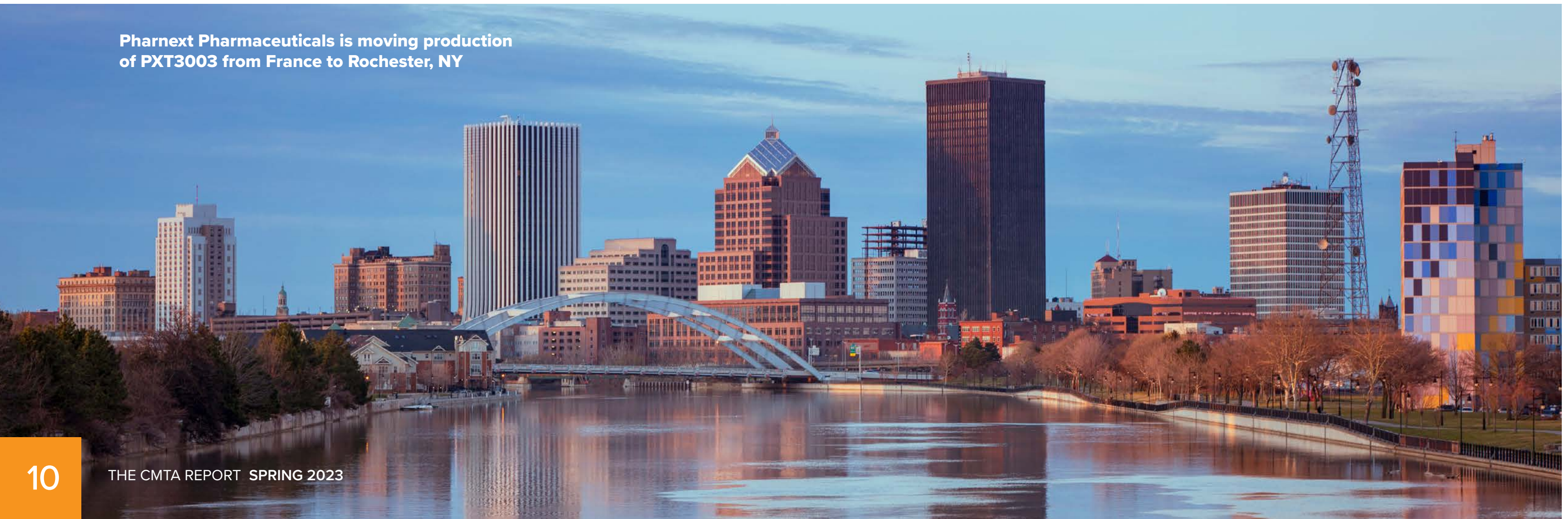
To date, almost 800 patients around the world have been enrolled in clinical trials of PXT3003, including:

- 80 patients in the Phase II clinical study;
- 323 patients in total enrolled in the first Phase III program (double-blind Phase III clinical study, the PLEO-CMT trial, followed by an ongoing open-label extension study, the PLEO-CMT-FU trial), including around 120 patients still on treatment with PXT3003 in the PLEO-CMT-FU trial;
- 387 patients in total enrolled in the second Phase III program (ongoing double-blind pivotal Phase III clinical study, the PREMIER trial, followed by an ongoing open-label extension study, the PREMIER-OLE trial), including around 40 patients already enrolled in the PREMIER-OLE trial on Jan. 1, 2023.

In May 2022, after five years of total trial time, data from the PLEO-CMT trial showed a sustained treatment benefit for patients treated with PXT3003 High Dose (the same dose tested in the PREMIER trial) with a good safety profile and continuous treatment effect as measured on the Overall Neuropathy Limitation Scale (ONLS), which evaluates patients’ functional motor disability.

Pharnext completed patient enrollment in the PREMIER trial in May 2022. Enrollment took place in 52 centers across the United States, Canada, Europe and Israel, with topline data expected to be announced in the fourth quarter of 2023. In September, the first patient who completed the PREMIER trial was enrolled in the PREMIER-OLE (Open Label Extension) trial. The decision to conduct the PREMIER-OLE trial was triggered by encouraging data from the open-label extension study of the first Phase III program, the PLEO-CMT-FU trial.

Brugière said the company looks forward “to generating additional long-term data from both our ongoing extension studies to confirm the potential safety and efficacy of PXT3003 for patients with CMT1A. For all of Pharnext’s teams, it is an ethical commitment to allow patients with CMT1A already enrolled in our Phase III studies to continue to have access to PXT3003 in open-label extension studies. These patients currently have no specific therapeutic options for CMT1A, and it is important to support them in their daily lives. We want to be a company truly focused on patients’ needs and support,” he concluded.


Pharnext Pharmaceuticals is moving production of PXT3003 from France to Rochester, NY

SKILLED NONPROFIT FUNDRAISER CRAIG ZELTSAR BRINGS EXPERTISE TO CMTA BOARD OF DIRECTORS

The CMTA welcomed Craig Zeltsar, principal and co-founder of NNE Marketing, LLC, to its Board of Directors in December 2022. NNE helps some of the nation’s top nonprofit organizations diversify their fundraising programs.

CMTA Board Chair Gilles Bouchard said Zeltsar brings to the CMTA amazing nonprofit experience combined with a true passion for helping the community. “It’s a privilege for us to welcome him to our team,” he added.

Zeltsar said, “I am honored to serve with such a distinguished group of people who are so passionate about helping people with CMT. I am eager to bring my experience working with dozens of nonprofits on their fundraising initiatives to a cause that is so personal to me and my family. I intend to bring the same perseverance to the CMTA that I bring to how I approach living with CMT, which is to not let it stop me from accomplishing anything.” Zeltsar and one of his two sons have CMT Type 1A, and the family has been actively involved in the CMT community for many years.

Zeltsar has 28 years of fundraising and membership experience, both on the agency and nonprofit side. He is an innovator who believes strongly in marrying analytics and creativity to develop a more donor-centric, customized experience for nonprofit constituents. He proudly advocates for his clients and the industry, positioning them for long-term success and growth.

Before forming NNE Marketing, Zeltsar was vice president of client services at THD. Prior to that, he worked in the fundraising and membership services group at Epsilon and at Mail Communications Inc. He began his fundraising career as the director of operations and community relations at Community Boating, Inc., in Boston.

Zeltsar frequently speaks on industry matters and recently served as vice chair of the ANA Nonprofit Federation Advisory Council. He holds a B.S. in business administration with a concentration in marketing from Bryant College in Rhode Island. In his spare time, Craig likes to ski, cook, bike and stay active outdoors.

SHIFT PHARMACEUTICALS AND CMTA PARTNER ON ASO TREATMENT FOR CMT1A

Shift Pharmaceuticals, a privately held company that is developing antisense oligonucleotides (ASOs) to treat a variety of genetic disorders, and the CMTA are collaborating on a project using ASOs to treat CMT1A. Type 1A is caused by a duplication in the PMP22 gene. ASOs are drugs that can alter RNA and reduce, restore or modify protein expression.

The collaboration takes advantage of the CMTA’s extensive suite of expert preclinical testing capabilities for companies like Shift Pharmaceuticals that want to evaluate the therapeutic potential of a drug candidate. The findings from the project will be critical in advancing research that will lead to clinical trials for CMT1A patients.

“Our entire team is excited to be working with the CMTA on the development of our lead candidate for CMT1A, SHC1A-012. Their expertise in preclinical development and funding natural history studies in this disease will greatly assist with the acceleration of our program to the preparation of the final Investigational New Drug Application and filing with the FDA,” said Steve O’Connor, CEO of Shift Pharmaceuticals. “Additionally, we feel strongly that their efforts in patient engagement will provide invaluable guidance in designing clinical outcomes to maximize the benefit for the entire CMT1A patient community.”

Shift PHARMACEUTICALS
Targeted therapeutics for neuromuscular disorders

WHEEL COVERS TURN WHEELCHAIRS INTO ART OBJECTS, CONVERSATION STARTERS

“If you can’t stand up, stand out” is the tagline for Izzy Wheels, a line of colorful covers for wheelchair wheels founded by Irish sisters Ailbhe and Izzy Keane. Their innovative idea earned them a place on the prestigious Forbes 30 under 30 list and partnerships with some of the world’s most famous brands, including Disney, Barbie and Hello Kitty.

The sisters, best friends all their lives, always loved fashion and playing dress-up together. Izzy, who was born with spina bifida and is paralyzed from her waist down, saw her wheelchair as a symbol of freedom but never felt it expressed her personality. As little girls the sisters decorated Izzy’s chair for special occasions. The decorated wheels gave Izzy a huge confidence boost, opening up positive conversations about her wheelchair.

Izzy Wheels was born in 2016 when Ailbhe (pronounced Alva), who was studying at The National College of Art and Design in Dublin, designed a range of removable wheel covers that matched her sister’s colorful style.

The sisters created a video showcasing their unique wheel cover designs, which went viral, racking up 17 million views in one week. Their brand exploded. In 2017, they began collaborating with other artists, designers and brands from around the world.


Izzy Wheels founders Izzy and Ailbhe Keane

In 2019, they teamed up with Barbie to create a range of designs for a new and inclusive wheelchair Fashionista Doll, which helps educate children about inclusivity. In 2020 they created two limited-edition wheel cover collections with Japan’s most famous cartoon character, Hello Kitty.

Finally, in 2021 they fulfilled a childhood dream and launched a collaboration with Disney and Marvel that included cartoon favorites from Mickey and Minnie, Winnie the Pooh, Olaf from Frozen and Spider-Man.

“Our mission with Izzy Wheels is to challenge negative associations with wheelchairs and let users celebrate their individuality by personalizing their source of independence,” Ailbhe said. “We want to show the world that wheelchairs can be so much more than a medical device. They can be a piece of artistic self-expression.”

Every pair of Izzy Wheels is specially made to order for each person at the sisters’ Dublin studio. The covers retail for approximately $162 and can be found at www.izzywheels.com.

SUPPORT THE CMTA IN THE FIGHT AGAINST THE PROGRESSIVE AND DEVASTATING EFFECTS OF CMT

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Complete and mail to the CMTA, PO Box 105, Glenolden, PA 19036

BE A FUNDRAISING FORCE MULTIPLIER
With Corporate Matching Programs

BY DAN MORGAN

Many companies include a corporate matching program as part of their employee benefits package. While these programs go by different names, they all work similarly: When an employee makes a charitable donation, the company will contribute a percentage of the donation to the same charity, up to a set amount per employee per year. Many members of the CMT community have used these programs to turbocharge their fundraising campaigns benefitting the CMTA.

For example, in my Cycle4CMT campaign in 2022, nearly one-third of the funds that I raised came through corporate matching programs used by my donors. I cycled with my brother, who is also a CMT1X patient, and one of my co-workers under the team name Morgan Brothers. What I learned about helping my donors increase the impact of their giving can help anyone with their fundraising efforts.

Getting donors to commit their hard-earned dollars in the first place takes the most legwork. But they also need to be aware of—and complete—additional steps to leverage this benefit, which means fundraisers need to do a little extra, making sure to:

- **Frame their mindset.** Corporate matching programs are a vital portion of an employee’s total compensation package aimed at attracting and retaining talent. In addition to supporting a good cause, you are encouraging your donors to take full advantage of the benefits their employers provide in exchange for their hard work and skills.
- **Bring awareness, empathetically.** Make sure that when soliciting and thanking donors, you bring up company matching programs. They may not be top of mind for your donors, if they’re even aware of their companies’ program. You can also go the extra mile and look into whether a donor’s employer has a matching program to further personalize your message. Many companies advertise this benefit on their website and in job postings. Most programs have an upper limit per employee, so be sensitive to the fact that your donors may exceed that threshold and need to prioritize their use of matching dollars across multiple causes.
- **Make it frictionless.** Research the exact parameters of your company’s program, create a step-by-step guide, and provide them to donors from your company with an offer to help. Share the details of your campaign with your HR contact and ask for any additional recommendations they have—there may be additional programs within your company’s corporate giving strategy that can support your efforts. For donors outside of your company, you can still highlight how simple it is and offer to help.


Fundraiser and biker Dan Morgan

The CMTA is a registered charity in third-party platforms used by many companies to administer these programs, requiring less input from the employee to complete the process. Additionally, the tool used for many CMTA campaigns includes a step in the donation workflow that allows a donor to search for their company’s match program, and in some cases, provides details on how to act. To learn more, visit: **www.cmtausa.org/employeematch.**

Dan Morgan is an IT enterprise architect based in New York City.

SCAN THIS QR CODE FOR MORE INFORMATION ABOUT MATCHING GIFTS. THE CMTA IS A PARTNER WITH DOUBLE THE DONATION, A COMPANY THAT MAKES IT EASY TO FIND OUT IF YOUR EMPLOYER WILL MATCH YOUR DONATION.

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Team McWeberSon Family Walk Proves Life Is Not a Spectator Sport

BY LISA WEBER

Three generations of my extended family live with CMT. My sister-in-law, Debi Weber, a branch leader in Madison, Wis., has been very active in the CMTA for years. Both her mother and her son also have CMT, and she is both a champion of and an inspiration to those affected by the disease. Because I live out of state, I was until recently unable to participate in any of Debi’s fundraising efforts in person. Last year, though, when I found out Debi’s daughter (my niece) would be visiting us during the Washington Branch’s Walk4CMT, I thought, “Let’s do this!”

After looking into the location (nearly two hours from our home at a school track), I wondered, can we create a team and walk closer to home, raising awareness and donations on the same day in a separate location?

I reached out to Mary Louie, the CMTA National Events Manager, and she was immediately on board. She helped me get a team signed up to do a “virtual walk” much closer to home under the Washington Walk4CMT and make shirts for our team.

As Team McWeberSon, we had nine walkers all from our family. We hiked more than five miles at Mount Rainier from Mowich Lake to Spray Park Falls, raising $2,150! The best part of the hike was that along the way, so many people asked us about our “Walk4CMT, One Step Closer to a Cure” matching T-shirts. We had great discussions with other hikers about why we were on this hike for our family members and the importance of raising awareness about CMT. Mission accomplished. We decided that this was just the first of many Team McWeberSon Walks4CMT.

Debi always says, “Life is not a spectator sport,” and we are happy we could embrace her motto.

WANT TO PLAN YOUR OWN Walk4CMT?
Visit cmtausa.org/walk4cmt

SPRING CLEANING TOOLS & TIPS
FOR ANYONE WITH LIMITED MOBILITY

BY BETH RUSH

Cleaning with limited mobility can make you feel like Hercules facing the Augean stables. You have a big, dirty job that seems insurmountable and a limited amount of time before you collapse.

The legendary man-god used irrigation to muck out the malodorous mess without getting his hands dirty. Simpler tools can help anyone with a disability knock those necessary chores out so they can focus on their health and healing.

Housekeeping creates more stress on disabled people, who already shoulder more than their fair share. However, it's one of the most essential tasks on their list. Regular cleaning and disinfecting are among the best ways to prevent infectious diseases, which can worsen chronic illness symptoms. It's vital for those with compromised immune systems.

Furthermore, everyday allergens and toxins can increase systemic inflammation, worsening symptoms. Filthy carpets can harbor E-coli bacteria; mold can cause a number of health concerns; and unchanged sheets can set off allergies that clog your nose, making sleep impossible.

Finally, a clean environment results in a psychological boost. Living with a chronic condition makes you feel like very little in your life remains within your control—your very body betrays you daily. Tidying your space restores your sense of agency.

Here are 10 cleaning tools that help with limited mobility, with bonus gadgets for disabled hands.

Beth Rush is the managing editor and content manager at Bodymind.com.

10 ESSENTIAL CLEANING TOOLS FOR THE DISABLED

You can make your housework a lot less onerous with these tools:

1. Steam Mop

It can be downright impossible to get on your hands and knees to scrub sticky spots off tile when you're cleaning with limited mobility. A steam mop keeps you upright, using the power of heat to dissolve that spilled splotch of jam.

2. Lightweight Vacuum

Dragging out a heavy vacuum is a hassle when every joint in your body screams with pain. Look for lightweight stick models such as those used in restaurants for quick cleanups.

3. Robot Vacuum

Better yet, invest in a robot vacuum. Pets can be valuable companions for people with disabilities, but their dander can increase allergies. These handy gadgets keep the fur down to a reasonable level.

4. Grabber

Standing on tiptoe can leave you dangerously wobbly. A grabber lets you reach things on high shelves and knock off the dust.

5. Cleaning Trolley

A traditional caddy might not work as a cleaning tool for the disabled if it's too awkward or bulky to carry with arthritic hands. A trolley lets you roll supplies from room to room.

6. Denture Tablets

Hard water stains can make your toilet look ancient. Drop in one of these tablets and let the fizz scrub away that rust.

7. Steam Microwave Cleaner

"Angry Mamas" are adorable. They're also ideal for loosening all that splattered gunk off the inside of your microwave so you can clean it with a single swipe.

8. Rubber Floor Mats

Does chopping broccoli make your hips and lower back protest loudly? Take a tip from restaurateurs who know that rubber standing pads are a must for chefs.

9. Scooter Stool or Electric Scooter

Extended periods of standing can leave you aching. A scooter stool or electric scooter gets you from place to place with less effort and lets you take a break between cleaning tasks.

10. Personal Protective Equipment

Rubber gloves protect your hands from germy surfaces. Furthermore, those COVID-19 masks that keep you safer at the grocery store also double as a cleaning tool for the disabled, reducing the number of chemical fumes you inhale.

FIVE GADGETS FOR DISABLED HANDS

You don't realize how much you rely on your hands until they hurt so badly you can't do simple daily tasks. Here are five bonus gadgets that people with disabilities often find handy.

1. Automatic Bottle and Jar Openers

What if you don't have a resident Hercules? These gadgets free you to grocery shop without considering the container holding your goods.

2. Padded Grips

Just as floor cushioning pads arthritic knees and hips, these grips do the same for your hands.

3. A Nimble (Soft Thimble)

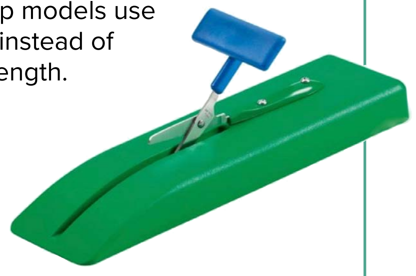
Does sorting your mail or a stack of paperwork drive you buggy because everything sticks together? This rubber thimble makes it a snap to separate sheets.

4. Handle Extenders

Do old-fashioned twist knobs stymie your wrists? Handle extenders let you open doors without pain.

5. Tabletop Scissors

Joint pain can make scissors impossible to manipulate. Tabletop models use a lever instead of grip strength.



THREE TIPS FOR CLEANING WITH LIMITED MOBILITY

You can have all the tools in the world, but you still need the right approach. Here are three tips for cleaning with limited mobility.

1. Automate

Smart home devices have made it possible to automate many cleaning tasks, like vacuuming. Make use of as many innovations as you can find. Even old-fashioned devices like The Clapper come in handy—especially if you just laid down after a strenuous bout of housecleaning and realize you forgot to turn out the light.

2. Schedule Small Jobs

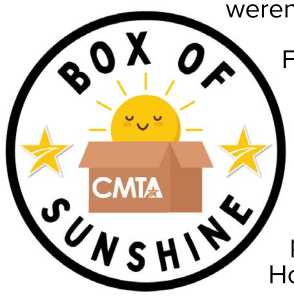
It's easy to get overwhelmed by how much there is to do and throw up your hands in defeat. Instead, schedule a few small tasks each day instead of one big weekly cleanup.

3. Clean As You Go

You can learn a lot from people who perform routine tasks like cooking for a living. When involved in projects that take multiple steps, clean as you go to avoid a huge mess at completion.

CMTA YOUTH MOVEMENT ON THE MOVE

The CMTA's Youth Movement hit the ground running in 2023. In February, they launched their "Boxes of Sunshine Project," one box aimed at providing support and resources for newly diagnosed youth and the other for youth preparing for CMT-related surgery. Both boxes contain links to CMTA youth resources, a copy of the youth book "Walk a Mile in My Braces," CMTA socks and stretchy bands. The surgery box also contains advice from kids who have already been through it. Requests can be made at bit.ly/3Y7u1Qr.



In April, the Third Annual CMTA Youth Dance4CMT will bring together youth and adults from around the world to raise money for CMT research. Evan Zeltsar, veteran Youth Council member and dance organizer, said, "There are a lot of exciting changes coming with this year's dance! Everyone will now be able to find (or host) a local dance in their area, keeping the same fun energy from past years and building even stronger in-person connections."

Camp Footprint camper Adeline Woods emphasized the importance of the dances to the youth community. She said, "I went to a school dance recently and found it couldn't compare to the dances we've had at Camp Footprint. At camp, you could dance any way you wanted with no judgment because we all understood why we weren't the most graceful."

Finally, the new COMPASS program for young adults aged 19 to 30 is gaining momentum. Before COMPASS, young people aged out of the youth program at the age of 18. However, as COMPASS co-leader Julia Beron explained, "When it was my time to graduate from the Youth Council, I knew my work with the CMTA wasn't done. We all wanted to continue the momentum and connectivity started in the Youth Movement. And the next step was young adults. Going through your 20s can be a super confusing, challenging time for anyone, and adding CMT to the mix—forget about it!"

Julia said, "We wanted to create a safe space for those in this age group where we can lean on one another for support and advice—so the CMTA started COMPASS." In just a short time the group has added more than 60 members.

COMPASS member Allie Dahlstrom said of the group, "It's really nice to know that I'm not alone. And it's also nice to meet people that have some similar issues as me. I look forward to our monthly meetings."

COMPASS
NAVIGATING CMT
AS A YOUNG ADULT

Topics at those meetings run the gamut from "Mental Health and CMT" to "Job Hunting with CMT." A full list of upcoming topics can be found at **cmtausa.org/get-involved/cmta-compass/.**

SUPPORT THE CMTA AND HONOR A LOVED ONE WITH AN IN HONOR/IN MEMORY GIFT

○ $50 ○ $100 ○ $250 ○ $500 ○ $1,000 ○ Other: $___________

○ **YES, I WANT TO BE AN INNERVATOR—**Please make my gift a monthly donation.

○ Please charge my credit card. ○ Visa ○ MasterCard ○ American Express

○ Check enclosed, payable to the Charcot-Marie-Tooth Association. Donate online at cmtausa.org/donate

To give the gift of stock, please call Jeana Sweeney at 814-269-1319

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Complete and mail to the CMTA, PO Box 105, Glenolden, PA 19036

HOW FAMILY SUPPORT
Carries Me On My CMT Journey

BY LISA WEINER

In December 1978, one month shy of my eighth birthday, my parents made the difficult decision to have me undergo a bilateral Achilles tendon-lengthening surgery to treat my extremely high arches and unstable, high-steppage gait. It would be another six years before the Stanford orthopedist treating me for scoliosis noticed all the common CMT symptoms and made the diagnosis.


Susan (center front) celebrating her birthday with her support system: boyfriend Gary Loeffler, mother Susan Weiner, father's partner Norm Dito and father Steve Weiner

The surgery was performed at Mills-Peninsula Hospital in San Mateo, Calif. I still remember the hospital and the terror I felt when I woke from the anesthesia. My mom, sitting in a chair at my bedside, smiled to reassure me that everything was going to be OK. While I was comforted by her presence, the stabbing pain overwhelmed my body. I couldn't speak to tell her that I loved her and that her being there meant everything to me.

The treating nurse shot me with morphine to dispel the pain, and I slept. Even through my screams and wails and concerns about whether the surgery had been the right choice, my mom never seemed to leave my side. Except for late at night, when I needed my bedpan changed or another morphine injection, I never felt alone.

My mom, dad, brother, grandma, grandpa, aunt and cousins all came to see me and sign my two full-length leg casts. They used brightly colored marking pens and drew funny pictures on them. Since there was plenty of lying down with legs elevated, there was ample time to inspect the artwork and count the signatures.

There were a few other young patients there, interacting with family members or friends after surgery and hospitalization. During one visit with my aunt and cousins, I remember being wheeled into the pediatric playroom. The room was filled with colorful toys, puzzles and games—it was like Disneyland to a 7-year-old girl. Everyone encouraged me, placing toys on my lap and initiating games.

I also remember being wheeled into the room of another young girl who was recuperating from orthopedic surgery. Talking and sharing our stories—and the sense of camaraderie that created—was like breathing in oxygen. Anyone in that situation needs to feel like they are not alone and scared lying in a hospital bed.

Nearly two decades later, at age 27, my right ankle desperately needed repair. Repeated ankle sprains had done their damage, rehabilitation had failed, and the pain had escalated. After months of physical therapy, an MRI finally confirmed significant ligament and tendon tears in my right ankle. At the time, I was sharing a third-floor walk-up apartment on one of the steepest hills of San Francisco's Cow Hollow district. My dad—who cared for his ailing elderly mother, ensured that senior family and friends had rides to synagogue or lifecycle events and devoted his career to consoling people grieving after the loss of a loved one—insisted I live with him for the first month post-surgery.

My dad lived in an apartment with 24-hour front desk security and, most importantly, an elevator. Though the apartment was spacious, it had only one bedroom, which my dad gave to me while he slept on his living room couch for four weeks. In the first week, he helped me to the toilet, arranged pillows to elevate my leg, brought me pain medication and prepared nourishing meals. After a week of recuperation, I returned to work. Each morning he helped me to the bathroom and brought me a washcloth, soap and towels so that I could sponge bathe myself. Afterward, I crawled back to the bedroom, lifted my heavy-casted leg up and began to dress in appropriate office attire of cast-accommodating loose pants or long skirts. Despite working long hours himself, Dad drove me to and from work for several weeks until a coworker who lived nearby offered to pick me up.

They say that disability and chronic medical conditions affect not only the afflicted but the entire family. I'm keenly aware of my great fortune in being born into a supportive, loving family. For more than 40 years, my family steps up to the plate each time I develop painful toe and finger infections; need additional bracing or mobility aids; and for every foot, ankle and hand surgery. My parents, my rocks, are always there listening and empathizing. It's their presence and action in times of crisis and despair that buoy my spirits and encourage my body and mind to recover, rehabilitate and thrive.

Adapted from Lisa Weiner's essay in "Moving Forward, Looking Back," published by the GAB Writer's Group and available on Amazon. Lisa is the cherished leader of the San Francisco Bay Area CMTA Branch.

Gambling on Humanity with an Invisible Disability

Lainie Ishbia, 52, was playing the slots in Las Vegas when the words no one ever wants to hear ripped through the casino: “Active shooter.” People began running frantically past her. Lainie froze, not in fear, though that was part of it. She froze because she has CMT and knew she wouldn’t be able to run away. For the first time in her life, she felt “utterly crippled and powerless.”

Thankfully, it was a false alarm. Erroneous reports of gunshots had triggered the warning. Had it been real, Lainie says now, she would have either been a sitting duck for a gunman, or she would have been trampled by strangers assuming she could run and keep up with the crowd.

Lainie, a disability speaker and blogger, told her story to a Jacksonville, Fla., audience in a TEDX Talk titled “Gambling on Humanity with an Invisible Disability.” Lifting her maxi-skirt to reveal her leg braces, Lainie put her disease in perspective: “Most of us have something invisible, whether it’s underneath our clothing or behind the mask we wear every day.”

“Imagine living with a secret that affects everything we do,” she said, adding, “Imagine how it would feel to never share your secret.” Revealing those secrets can be a huge gamble, she said, and not the fun slot-machine kind. “It’s like putting all the cards you’ve been dealt with in life face up on the table—and then betting it all on humanity,” she added.

Pointing out that 61 million adults in the United States live with disabilities, Lainie told the audience, “You’ve probably seen us rolling in wheelchairs, walking with service dogs, using a cane or maybe even running a marathon with missing limbs.” But 80 percent of disabilities are invisible.

Odds are that most people will join this group of people with disabilities at some point, she said, adding “We have rolling admissions and we’re the most inclusive club in town.” Invisible disabilities include everything from MS to diabetes to vision loss and chronic pain to lupus to ADD.

> “Most of us have something invisible, whether it’s underneath our clothing or behind the mask we wear every day.”

Lainie kept her disability a secret most of her life. Growing up, she longed to be like the models in Seventeen magazine. As her condition worsened, she took to making up answers to the question “What’s wrong with you?” claiming that she’d had a skiing or rock-climbing accident.

Those unwanted questions were her introduction to ableism in our society, she said. Ableism can be overt—as when corporations don’t comply with the Americans with Disabilities Act, or it can be unintentional, as when people make comments like, “You’re too pretty to be in a wheelchair” or “Have you tried keto? It cured my aunt’s MS.”


Laine turned her harrowing experience into a TedTalk. You can watch it on YouTube.

Being met with judgment and ignorance can be triggering, Lainie said, adding, “Given the stigma, why would anyone choose to disclose their disability?”

She finally disclosed her disability at the age of 42, starting a website called Trend-Able and posting pictures of her legs and braces online. Her gamble paid off: Not only was it cathartic, but it actually strengthened her relationships.

In order to erase the stigma of disability, more people need to share their experiences, Lainie said. If everyone with a disability shared their gifts with the world instead of hiding, it would trigger a jackpot, she added, and a lifetime payout that could benefit everyone.

You can read more by and about Lainie in her blog at Trend-Able.com. Her TedTalk is available at https://youtu.be/-dneOuefRyU. She offers “EmBrace It” workshops to promote sensitivity and educate people about disabilities in the workplace.


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challenge their limits and
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no matter the obstacle!

– Aika, Doctor of Physical Therapy and 2- time Paraclimbing World Champion (RP3)

My Invisible Disease

BY CHLOE ALPERIN

Everyone has moments that change their view of life. Mine came when I was diagnosed with CMT, a vile genetic disease that causes problems with the nerves and muscles.

Looking back, I wondered why my toes were going numb. Eventually, I couldn’t tolerate it anymore and told my parents. They scheduled an appointment with the doctor who told me that I would “grow out of it.”

Then COVID struck. I got my first vaccine with no problems, but things went downhill after the second. I was on the beach with my mom and we were getting ready to leave. I stood up and couldn’t feel my feet at all. Even though I couldn’t feel them, it was excruciatingly uncomfortable. My parents took me back to the doctor, who ordered genetic testing. I wasn’t expecting anything to show up, so when they called me in to tell me I had CMT, I was astounded.

My doctor went over what this disease was and what symptoms I could expect, then scheduled appointments with a clinical team, including a neurologist, genetic counselor and physical and occupational therapists to determine how far the disease had progressed.

While working on exercises to maintain my strength, I had good days and bad days. Sometimes I felt as if I couldn’t do anything, and other times I felt invincible. The emotional rollercoaster of life! My parents supported me through the whole process, and my mom even helps me with therapy exercises since she is a therapist. Eventually, my mood swings diminished.

I experience blood pooling in my legs and burning sensations from the nerves. I sometimes have numbness in my feet and weakness throughout my whole body. I also experience exhaustion after a simple task. I have to sleep with braces to support my feet because of my ankle weakness. I may not be able to do things a normal person is able to do, but I try. I will keep trying and working through these hardships. Who knows what my future holds? I might be fully functional, or I might not be able to walk without assistance and have to depend on a wheelchair.

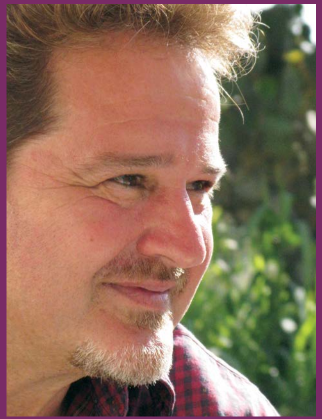

Chloe says the weight of CMT “lies heavy” on her shoulders.

> “I may not be able to do things a normal person is able to do, but I try. I will keep trying and working through these hardships. ”

The weight of this invisible disease lies heavy on my shoulders. People can’t tell that I feel burning, numbness, weakness and fatigue every day. I look completely normal and healthy and might appear lazy to those who can’t see the true colors of my disease. In the meantime, I have to stay strong and keep friends and family by my side, supporting me through life’s challenges. Until then, I wake up every day hoping for a cure.

Chloe, 15, is in the 10th grade and lives in Norfolk, Va. She has CMT1A.

WHAT'S ON YOUR MIND? ASK DAVID.



David Tannenbaum answers questions from readers in his column "What's On Your Mind? Ask David" in The CMTA Report. *David has an LCSW degree and has been a psychotherapist in New York City for the past 30 years, specializing in helping others with the task of growing emotionally and spiritually through physical challenges. "My CMT has been my greatest challenge and my best teacher in life," says David.*

Dear David,

I am a 64-year-old man with CMT1A. I recently retired from my job as a data scientist, which didn't require a lot of physical movement and gave me purpose and a group of co-workers I really enjoyed. I've always needed to feel useful, whether by shoveling a neighbor's driveway or otherwise lending a helpful hand. I'm a big guy and enjoyed my ability to help others. Unfortunately, my hands don't work well these days, and forget about shoveling anything. I tried to help a neighbor with her groceries the other day and tripped, dropping everything including her eggs. She looked at me with pity and all I could do was apologize for my clumsiness.

I've never talked about my CMT to my neighbors, but I suspect they know something is not quite right. My wife encourages me to speak to them but I'm a really private person. Meanwhile, I find myself avoiding them more and more and feeling down. What should I do?

David Replies:

Trying to pass as someone without issues causes a lot of anxiety. Trying to pretend to be someone you're not creates distance between you and the world. It's exhausting to worry about what others think, and it can harm your emotional and physical well-being. You sound like a great guy who is hiding a major part of your life. Perhaps it feels like being honest with others will make them see you in a negative light. My experience has been just the opposite. Being honest about your situation generally creates openness and gives others permission to be honest as well. This kind of authenticity creates real connections.

About 10 years ago, I tried to help an elderly woman up her stairs and ended up pulling her down with me. I felt so humiliated and inadequate. I was the opposite of the "knight in shining armor." I realized that the days of seeing myself as a rescuer were over. I didn't realize at the time how much of my self-esteem was tied up in this image. Accepting my limitations was not easy and I went through yet another layer of the grieving process. Feeling sorry for ourselves, sadness and even anger are all normal responses to acknowledging our physical limitations. Yet accepting our CMT ultimately brings liberation and peace. Don't resist these uncomfortable feelings, but at the same time, don't wallow in them. Feel your feelings and then allow them to leave on their own.

Once my grieving began to subside, I began to see that there were so many ways to be helpful that had nothing to do with my physical strength. Having compassion for what we go through on a daily basis is vital. Compassion for one's struggles is very different than self-pity. Self-pity is more about self-loathing while compassion is self-love. Our suffering helps us develop true empathy for other people's emotional or physical pain and is the most beautiful quality we possess.

We often forget that a kind word or a smile is an action that has enormous power to help someone feel good and make their day. There has been some interesting research recently on the power of kindness and how simple acts of kindness have measurable positive effects on both the giver and receiver. One study found that these small acts of kindness actually lower levels of depression and anxiety. Simply paying real attention and truly listening to others is what most people crave. We are all very capable of doing this. With computers, there is no excuse for not staying connected to friends and family. Some people, including me, believe that simply being home and sending out loving thoughts to the world can raise the consciousness of our planet. What could be more helpful and purposeful than that!

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BRANCH NOTES

SAN FRANCISCO, CA

New branch leader Lisa Weiner held her first branch meeting on Jan. 28—the first in-person gathering in more than three years. Both new and longtime branch members (22 altogether) attended, including a few who drove 90 miles to attend. Guest speaker Beth Weiss, a certified occupational therapist and certified hand therapist for 45 years at San Mateo's Mills-Peninsula Medical Center, joined the meeting. Beth's presentation included a show-and-tell of numerous adaptive aids for those who have hand weakness and fine motor skill challenges. She gave each person their own square piece of grippy drawer liner (a multipurpose aid to help people grip things easier so they can, e.g., open a tight jar). She also gave out lamp turners and rubber pen or pencil aid/attachments. Beth shared numerous strategies for best practices on using the hands to conserve energy, prevent injury and maintain good posture.


San Francisco


Hartford

HARTFORD, CT

Nine Hartford Branch members and families met on Sept. 24, 2022, for an Awareness Month breakfast. Over eggs, French toast and pancakes, they talked about the difficulties of getting good shoes that fit with AFOs, enhance balance and minimize blisters caused by foot deformities. Members shared personal experiences with neurologists, Centers of Excellence and orthotics and agreed that it was great to meet in person again.

DESTIN, FL

The Destin Branch's last meeting included conversations on bracing and an update on upcoming CMTA events. Chance Gray, mid-South district manager for Allard USA, spoke about Allard's products for foot drop and related complications from CMT and demonstrated several styles of braces. CMTA Community Outreach Director Laurel Richardson shared information on upcoming CMTA activities and events and asked for input on how CMTA might better serve the community.

HAWAII

Nine members of the Hawaii Branch met in January to catch up with each other and share local resources. They welcomed new branch co-leader Monica Rocabado, who planned the meeting and will help with upcoming meeting planning. The group also discussed relevant topics such as surgery, shoes and tips for adapting to CMT progression.

CHICAGO, IL

The Chicago Branch welcomed Dr. Daniela Menichella, director of the CMTA Center of Excellence at Northwestern, to its latest meeting. She talked about how a neurologist treats neurological pain, which is what most people with CMT complain about. Menichella explained how physicians listen to their patients to find out what kinds of pain they are experiencing and then use scientific research and protocol to choose the first line of treatment.

GREATER MINNEAPOLIS, MN


Dr. Walk

The Greater Minneapolis Branch met for its inaugural meeting last August with new branch leaders Lynn Anne Groebner and Angela Christensen. Attendees introduced themselves and shared their CMT connections. They discussed fundraising for 2023 and plans for future branch meetings. Branch members met again on Dec. 3, 2022, both online and in person. Dr. David Walk, a neurologist at the University of Minnesota/Fairview and the head of the Minneapolis CMTA Center of Excellence, provided a great review of CMT, including history and details about the different types.

NEW MEXICO

The New Mexico Branch met virtually Aug. 6, 2022, welcoming guest speaker Michael Boczkiewicz, certified orthotist at Advanced Prosthetics and Orthotics. Mike shared insurance information related to orthotics and showed just a few of the many available types of bracing options.

SYRACUSE, NY

The Syracuse Branch met virtually on Aug. 22, 2022. Eight members talked about practical ways to stay positive while living with CMT and the importance of meeting with the medical professional team at the Center of Excellence at Strong Hospital in Rochester, NY. They also talked about the importance of AFOs, the types of shoes that make walking comfortable and the importance of staying involved in the CMTA community.

WESTCHESTER, NY

The Westchester Branch welcomed guest speaker Dr. Florian Thomas, board-certified neurologist and director of the CMTA Center of Excellence at Hackensack University Medical Center to its meeting Sept. 10, 2022. He gave an excellent presentation and answered questions in an easily accessible way. He also shared information on what patients can expect when coming to the CMT clinic.

RESEARCH TRIANGLE AREA, NC

The Research Triangle Area Branch met on Nov. 5, 2022, via Zoom to discuss occupational therapy and help for CMT hands. They viewed a video from the CMTA website by Valery Hanks, OT, at Vanderbilt University Medical Center and CMTA Advisory Board member. Valery then joined the meeting to answer questions about CMT and its effects on hands.

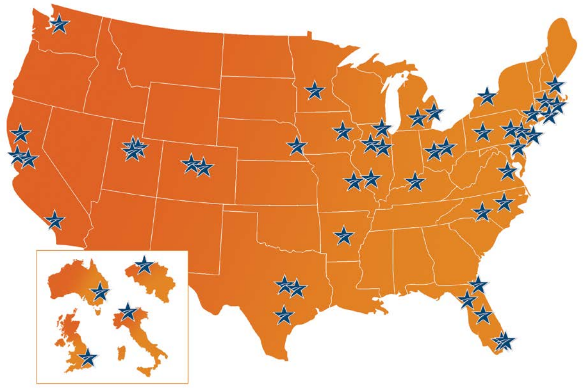
PORTLAND, OR

Led by Jacquelene Bennett, the Portland Branch is up and running again. The branch met virtually in October with a social hour where members shared their stories, braces and balance issues, holiday plans and exercise/mobility issues. It was a wonderful opportunity for the Portland Branch to get to know its new members. The branch has several meetings planned for 2023, both in person and virtual.


Portland

CMTA CENTERS OF EXCELLENCE

CMTA CENTERS OF EXCELLENCE roughly correspond to the 21 international sites that make up the NIH Inherited Neuropathy Consortium (INC)—a group of academic medical centers, patient support organizations and clinical research resources sponsored in part by the CMTA. The centers are becoming even more important as the CMTA begins clinical trials, which depend on how much we know about the "natural history" of CMT—how different types of CMT progress over time and whether novel medications are slowing the course of the disease. Much of that information will be supplied by the Centers of Excellence.



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LITTLE ROCK (PEDIATRIC)
Arkansas Children's Hospital
Clinical Director:
Aravindhan Veerapandiyan, MD
Appts: 501-364-1850

CALIFORNIA

LOS ANGELES (ADULT & PEDIATRIC)
Cedars-Sinai Medical Center
Clinical Director: Richard A. Lewis, MD
Appts: 310-423-4268

PALO ALTO (PEDIATRIC)
Stanford Children's Health*
Clinical Directors: John Day, MD, PhD, and Ana Tesi Rocha, MD
Appts: 650-723-0993

PALO ALTO (ADULT)
Stanford Neuromuscular Program*
Clinical Director: John Day, MD, PhD
Appts: 650-723-6469

SAN FRANCISCO (PEDIATRIC)
University of California, San Francisco
Clinical Director: Alex Fay, MD, PhD
Appts: 415-353-7596

COLORADO

AURORA (ADULT)
University of Colorado*
Clinical Director: Vera Fridman, MD
Appts: 720-848-2080

AURORA (PEDIATRIC)
Children's Hospital Colorado
Clinical Director: Michele Yang, MD
Appts: Alison Ballard, 720-777-3907

CONNECTICUT

FARMINGTON (PEDIATRIC)
Connecticut Children's Medical Center, Farmington*
Clinical Director: Gyula Acsadi, MD, PhD
Appts: Nanci Stolgitis, RN, 860-837-7500

NEW BRITAIN (ADULT & PEDIATRIC)
Hospital for Special Care
Clinical Director: Kevin J. Felice, DO
Appts: Sharon McDermott, 860-612-6305

NEW HAVEN (ADULT)
Yale School of Medicine
Clinical Director: Sasha Zivkovic, MD
Appts: 203-785-4085

FLORIDA

ORLANDO (PEDIATRIC)
Nemours Children's Hospital*
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Omer Abdul Hamid, MD
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GAINESVILLE (ADULT & PEDIATRIC)
University of Florida
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James Wymer, MD, PhD
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JACKSONVILLE (ADULT)
Mayo Clinic Florida
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University of Miami*
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University of Illinois at Chicago
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Rush University
Clinical Director: Ryan D. Jacobson, MD
Appts: Sherri Taylor-Kennedy, 312-942-4500

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HSHS St. Elizabeth's Hospital
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Raghav Govindarajan, MD
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University of Iowa Hospitals & Clinics*
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University of Louisville
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BALTIMORE (ADULT & PEDIATRIC)
Johns Hopkins University*
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BOSTON (ADULT & PEDIATRIC)
Massachusetts General Hospital
Clinical Director:
Reza Seyedsadjadi, MD
Appts: Tamika Scott, 617-726-3642

MICHIGAN

DETROIT (ADULT)
Wayne State University School of Medicine and Detroit Medical Center*
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ANN ARBOR (ADULT & PEDIATRIC)
University of Michigan
Clinical Director: Gary Gallagher, MD
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MINNESOTA

MAPLE GROVE (ADULT)
University of Minnesota*
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Appts: 763-898-1000

MISSOURI

ST. LOUIS (ADULT & PEDIATRIC)
Washington University School of Medicine
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OMAHA (ADULTS)
Chi Health Clinic Neurology Institute
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NEW JERSEY

HACKENSACK (ADULT & PEDIATRIC)
Hackensack University Medical Center
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NEW YORK

ROCHESTER (ADULT & PEDIATRIC)
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NORTH CAROLINA

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Atrium Health Neurosciences Institute
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OHIO

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Nationwide Children's Hospital
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COLUMBUS (ADULT & PEDIATRIC)
Ohio State University, Wexner Medical Center*
Clinical Director:
Clinical Director: Michael Isfort, MD
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PENNSYLVANIA

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Hospital of the University of Pennsylvania*
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Shana Millner, 215-662-3606
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Pooja Patel, 215-898-0180

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Children's Hospital of Pittsburgh
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TEXAS

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DALLAS (PEDIATRIC)
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BEDFORD (ADULT 16+)
Kane Hall Barry Neurology
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HOUSTON (ADULT)
Houston Methodist Hospital
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UTAH

SALT LAKE CITY (ADULT & PEDIATRIC)
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SALT LAKE CITY (PEDIATRIC)
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SALT LAKE CITY (PEDIATRIC)
Shriner's Hospital
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WASHINGTON

SEATTLE (ADULT)
University of Washington Medical Center*
Clinical Director: Michael Weiss, MD
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WASHINGTON, DC

(PEDIATRIC TO AGE 21)
Children's National Hospital
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AUSTRALIA

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*** Denotes the CMTA Centers of Excellence that are included in the National Institutes of Health Inherited Neuropathy Consortium (INC). The INC is co-sponsored by the CMTA and the MDA. Worldwide more than 10,000 patients with CMT have been enrolled in protocols, and their data, de-identified to protect patient privacy, is housed in a common repository. As a result of this collaboration, a new CMT evaluation scale for children has been established, along with a new evaluation system for adults, and an infrastructure has been developed to perform natural history studies and clinical trials for CMT.**

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ALL CMTA BRANCHES CAN BE ACCESSED ONLINE AT www.cmtausa.org/branches

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CMT PATIENT MEDICATION ALERT:

Definite high risk (including asymptomatic CMT):

Taxols (paclitaxel, docetaxel, cabazitaxel)
Vinca alkaloids (Vincristine)

Moderate to significant risk:

Amiodarone (Cordarone)
Arsenic Trioxide (Trisenox)
Bortezomib (Velcade)
Brentuximab Vedotin (Adcetris)
Cetuximab (Erbitux)
Cisplatin and Oxaliplatin
Colchicine (extended use)
Dapsone
Didanosine (ddI, Videx)
Dichloroacetate
Disulfiram (Antabuse)
Eribulin (Halaven)
Fluoroquinolones
Gold salts
Ipilimumab (Yervoy)
Ixabepilone (Ixempra)
Lefluonamide (Arava)
Lenalidomide (Revlimid)
Metronidazole/Misonidazole (extended use)
Nitrofurantoin (Macrodantin, Furadantin, Macrobid)
Nitrous oxide (inhalation abuse)
Nivolumab (Opdivo)
Pembrolizumab (Keytruda)
Perhexiline (not used in US)
Pomalidomide (Pomalyst)
Pyridoxine (mega dose of Vitamin B6)
Stavudine (d4T, Zerit)
Suramin
Thalidomide
Zalcitabine (ddC, Hivid)

Uncertain or minor risk:

5-Fluouracil
Adriamycin
Almitrine (not in US)
Chloroquine
Cytarabine (high dose)
Ethambutol
Etoposide (VP-16)
Gemcitabine
Griseofulvin
Hexamethylmelamine
Hydralazine
Ifosfamide
Infliximab
Isoniazid (INH)
Lansoprazole (Prevacid)
Mefloquine
Omeprazole (Prilosec)
Penicillamine
Phenytoin (Dilantin)
Podophyllin resin
Sertraline (Zoloft)
Statins
Tacrolimus (FK506, Prograf)
Zimeldine (not in US)
a-Interferon

Negligible or doubtful risk:

Allopurinol
Amitriptyline
Chloramphenicol
Chlorprothixene
Cimetidine
Clioquinol
Clofibrate
Cyclosporin A
Enalapril
Glutethimide
Lithium
Phenelzine
Propafenone
Sulfonamides
Sulfasalazine

The medications listed above are potentially toxic to CMT patients. Vincristine has been proven hazardous and should be avoided by all CMT patients, including those with no symptoms. Taxols also pose a high risk to people with CMT. The remainder of the medications listed above present varying degrees of potential risk for worsening CMT neuropathy. Before taking any medication or changing medication, all CMT patients should make sure the treating physician is fully aware of their medical condition.

WHAT IS **CMT?**

More than 3 million people worldwide have CMT. It is one of the most commonly inherited nerve disorders and affects the motor and sensory nerves.

CMT is slowly progressive, causing the loss of muscle function and/or sensation in the lower legs and feet, as well as hands and arms.

People in **all ethnic groups may be affected by CMT.**

CMT is genetic, but it can also develop as a new, spontaneous mutation.

CMT can vary greatly in severity, even within the same family.

CMT causes structural deformities such as high-arched or very flat feet, hammertoes, hand contractures, scoliosis (spinal curvature) and kyphosis (rounded back).

CMT can also cause foot drop, poor balance, cold extremities, cramps, nerve, muscle and joint pain, altered reflexes, fatigue, tremor, sleep apnea, hearing loss and breathing difficulties.

CMT rarely affects life expectancy.

Some medications are neurotoxic and pose a high risk to people with CMT, notably Vincristine and Taxols. See full list (at left) of medications that may pose a risk.

More than 100 different genetic causes of CMT have been identified.

Many types of CMT can be determined by genetic testing. Please consult with a genetic counselor (www.nsgc.org) or your physician for more information.

Although there are no drug treatments for CMT, a healthy diet, moderate exercise, physical and/or occupational therapy, leg braces or orthopedic surgery may help maintain mobility and function.

The CMTA-STAR research program and extensive partnerships with pharmaceutical companies are driving remarkable progress toward delivering treatments for CMT, bringing us closer to a world without CMT.